

Stem Cell Agency Invests in New Immunotherapy Approach to HIV, Plus Promising Projects Targeting Blindness and Leukemia

Posted: October 18, 2018

Oakland, CA – While we have made great progress in developing therapies that control the AIDS virus, HIV/AIDS remains a chronic condition and HIV medicines themselves can give rise to a new set of medical issues. That's why the Board of the California Institute for Regenerative Medicine (CIRM) today awarded \$3.8 million to a team from City of Hope to develop an HIV immunotherapy.

The City of Hope team, led by Xiuli Wang, is developing a chimeric antigen receptor T cell or CAR-T that will enable them to target and kill HIV-infected cells. These CAR-T cells are designed to respond to a cytomegalovirus (CMV) vaccine to expand on demand to battle residual HIV as required.

Jeff Sheehy, a CIRM Board member and patient advocate for HIV/AIDS, says there is a real need for a new approach. "With 37 million people worldwide living with HIV, including one million Americans, a single treatment that cures is desperately needed. An exciting feature of this approach is the way it is combined with the CMV vaccine. Making CAR-T therapies safer and more efficient would not only help produce a new HIV treatment but would help with CAR-T cancer therapies and could facilitate CAR-T therapies for other diseases."

This is a late stage pre-clinical program with a goal of developing the cell therapy and getting the data needed to apply to the Food and Drug Administration (FDA) for permission to start a clinical trial.

The Board also approved three projects under its Translation Research Program, this is promising research that is building on basic scientific studies to hopefully create new therapies.

- \$5.068 million to University of California at Los Angeles' Steven Schwartz to use a patient's own adult cells to develop a treatment for diseases of the retina that can lead to blindness
- \$4.17 million to Karin Gaensler at the University of California at San Francisco to use a leukemia patient's own cells to develop a vaccine that will stimulate their immune system to attack and destroy leukemia stem cells
- Almost \$4.24 million to Stanford's Ted Leng to develop an off-the-shelf treatment for age-related macular degeneration (AMD), the leading cause of vision loss in the elderly.

The Board also approved funding for seven projects in the Discovery Quest Program. The Quest program promotes the discovery of promising new stem cell-based technologies that will be ready to move to the next level, the translational category, within two years, with an ultimate goal of improving patient care.

Application	Title	Institution	CIRM Committed Funding
DISC2-10979	Universal Pluripotent Liver Failure Therapy (UPLIFT)	Children's Hospital of Los Angeles	\$1,297,512
DISC2-11105	Pluripotent stem cell-derived bladder epithelial progenitors for definitive cell replacement therapy of bladder cancer	Stanford	\$1,415,016

DISC2-10973	Small Molecule Proteostasis Regulators to Treat Photoreceptor Diseases	U.C. San Diego	\$1,160,648
DISC2-11070	Drug Development for Autism Spectrum Disorder Using Human Patient iPSCs	Scripps	\$1,827,576
DISC2-11183	A screen for drugs to protect against chemotherapy-induced hearing loss, using sensory hair cells derived by direct lineage reprogramming from hiPSCs	University of Southern California	\$833,971
DISC2-11199	Modulation of the Wnt pathway to restore inner ear function	Stanford	\$1,394,870
DISC2-11109	<i>Regenerative Thymic Tissues as Curative Cell Therapy for Patients with 22q11 Deletion Syndrome</i>	Stanford	\$1,415,016

Finally, the Board approved the Agency's 2019 research budget. Given CIRM's new partnership with the National Heart, Lung, Blood Institute (NHLBI) to accelerate promising therapies that could help people with Sickle Cell Disease (SCD) the Agency is proposing to set aside \$30 million in funding for this program.

"I am deeply grateful for organizations like CIRM and NHLBI that do vital work every day to help people struggling with Sickle Cell Disease," said Congresswoman Barbara Lee (D-CA 13th District). "As a member of the House Appropriations Subcommittee on Labor, Health and Human Services, and Education, I know well the importance of this work. This innovative partnership between CIRM and NHLBI is an encouraging sign of progress, and I applaud both organizations for their tireless work to cure Sickle Cell Disease."

Under the agreement CIRM and the NHLBI will coordinate efforts to identify and co-fund promising therapies targeting SCD. Programs that are ready to start an IND-enabling or clinical trial project for sickle cell can apply to CIRM for funding from both agencies. CIRM will share application information with the NHLBI and CIRM's Grants Working Group (GWG) – an independent panel of experts which reviews the scientific merits of applications – will review the applications and make recommendations. The NHLBI will then quickly decide if it wants to partner with CIRM on co-funding the project and if the CIRM governing Board approves the project for funding, the two organizations will agree on a cost-sharing partnership for the clinical trial. CIRM will then set the milestones and manage the single CIRM award and all monitoring of the project.

"This is an extraordinary opportunity to create a first-of-its-kind partnership with the NHLBI to accelerate the development of curative cell and gene treatments for patients suffering with Sickle Cell Disease" says Maria T. Millan, MD, President & CEO of CIRM. "This allows us to multiply the impact each dollar has to find relief for children and adults who battle with this life-threatening, disabling condition that results in a dramatically shortened lifespan. We are pleased to be able to leverage CIRM's acceleration model, expertise and infrastructure to partner with the NHLBI to find a cure for this condition that afflicts 100,000 Americans and millions around the globe."

The budget for 2019 is:

Program type	2019
CLIN1 & 2	\$93 million
CLIN1& 2 Sickle Cell Disease	\$30 million
TRANSLATIONAL	\$20 million
DISCOVER	\$0
EDUCATION	\$600K

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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